Non-interventional study for long term documentation of treatment with Vihuma in patients with hemophilia A (Biotest NIS-019)

First published: 26/04/2017

Last updated: 02/07/2024

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/33454

EU PAS number

EUPAS18757

Study ID

33454

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Germany
- [ ] Switzerland

Study description

The non-interventional Study has been cancelled due to the lack of participating sites and patient numbers at this time. No sites or patients have been recruited.Vihuma is a recombinant factor VIII (FVIII) produced in the human cell line HEK 293F (Human Embryonic Kidney Cells). Vihuma is approved for treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency). Vihuma can be used for all age groups. Details are given in the SPC and German Fachinformation of Vihuma.Hemophilia A is an inherited, chronic bleeding disorder and patients have to be treated lifelong with FVIII concentrates. Most children and adolescents are treated prophylactically in industrialized countries. Prophylaxis has the goal to avoid bleedings, in order to guarantee the patient a high quality of life (QoL).This will be the first NIS allowing direct comparison of treatment with a recombinant (Vihuma) and plasmatic factor VIII product (Haemoctin SDH, Biotest NIS-016), since data obtained in this two NIS are very similar.

Study status

Finalised

Research institutions and networks

Institutions

Biotest

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Institution

Multiple centres: 18 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Wolfgang Miesbach

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 23/02/2017

Actual: 02/03/2017

Study start date
Planned: 02/01/2020

Actual: 31/12/2019

Data analysis start date
Planned: 30/06/2025

Actual: 31/12/2019

Date of final study report
Planned: 31/12/2025

Actual: 31/12/2019

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biotest AG

Study protocol

2017-04-12 FVIII recombinant NIS Observation Plan 1.0.pdf(215.44 KB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Data collection methods:

Primary data collection

Main study objective:

With this NIS long-term data for the effectiveness in bleeding prevention and on QoL will be generated.What is the dose and frequency of Vihuma in prophylaxis?Is it possible to reduce the dose or extend the frequency of applications compared to previously used factor VIII products?What are the factors influencing the risk of bleeding over the time of treatment?

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Prospective, single arm study

Study drug and medical condition

Name of medicine

VIHUMA

Medical condition to be studied

Factor VIII deficiency

Population studied

Short description of the study population

Patients with hemophilia A treated with Vihuma recombinant factor VIII (FVIII) at home and, in some exceptional cases, in the clinic or a local doctor's practice.
Patients with following criteria were included:
1. Treatment in accordance with the SPC for Vihuma
2. Children of all ages and adult patients with FVIII deficiency (previously treated and previously untreated patients)
3. Written informed consent to allow data collection and data transfer to third party

Age groups

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Special population of interest

Immunocompromised

Estimated number of subjects

60

Study design details

Outcomes

Annual bleeding rate defined as episodes per year in patients with Vihuma treatment, differentiated by prophylaxis and on demand treatmentDose and frequency of Vihuma applications in comparison to previously used factor VIII products, AE and subsequent suspected ADR (AE assessed as causally related with Vihuma treatment)AE with bleeding = AE of special interest (AESI) with extended bleeding documentation, for e.g. if the duration and severity of the bleeding is within the situation as expected or unexpectedOccurrence and characterization of FVIII inhibitors to Vihuma

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Data analysis plan

All analyses will be performed in an exploratory sense. Data will be analyzed using descriptive statistics. For continuous variables, mean, standard deviation,

minimum, maximum, median, 25% and 75% percentiles will be presented. Qualitative and categorical variables will be presented by means of absolute and relative frequencies. A medical evaluation of the findings will be performed. Details of analysis will be described in a statistical analysis plan.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No